

## CDER Breakthrough Therapy Designation Approvals

As of December 31, 2015

Application Number	Submission Type and Number	Proprietary Name	Established Name	Applicant	Approval Date	Use
BLA 125514	SUPPLEMENT-6	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	18-Dec-2015	Treatment of patients with unresectable or metastatic melanoma.
NDA 208434	ORIGINAL-1	ALECENSA	ALECTINIB	HOFFMANN-LA ROCHE INC	11-Dec-2015	Treatment of patients with anaplastic lymphoma kinase (ALK)-positive metastatic non-small cell lung cancer (NSCLC), who have progressed on or are intolerant to crizotinib
BLA 125561	ORIGINAL-1	KANUMATM	SEBELIPASE ALFA	SYNAGEVA BIOPHARMA CORP	08-Dec-2015	Treatment of patients with a diagnosis of lysosomal acid lipase (LAL) deficiency
BLA 761035	ORIGINAL-1	EMPLICITI	ELOTUZUMAB	BRISTOL-MYERS SQUIBB COMPANY	30-Nov-2015	Treatment of patients with multiple myeloma who have received one to three prior therapies
BLA 125554	SUPPLEMENT-12	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	23-Nov-2015	Treatment of advanced renal cell carcinoma patients who have received prior antiangiogenic therapy
BLA 761036	ORIGINAL-1	DARZALEX	DARATUMUMAB	JANSSEN BIOTECH INC	16-Nov-2015	Treatment of patients with multiple myeloma who have received at least 3 prior lines of therapy including a proteasome inhibitor and an immunomodulatory agent or are double refractory to a proteasome inhibitor and an immunomodulatory agent
NDA 208065	ORIGINAL-1	TAGRISSO	OSIMERTINIB	ASTRAZENECA PHARMACEUTICALS LP	13-Nov-2015	Treatment of patients with metastatic epidermal growth factor receptor (EGFR) T790M mutation-positive-non-small-cell lung cancer (NSCLC), as detected by an FDA approved test, who have progressed on or after EGFR TKI therapy
BLA 125513	ORIGINAL-1	STRENSIQ	ASFOTASE ALFA	ALEXION PHARMACEUTICALS INC	23-Oct-2015	Treatment of patients with perinatal/infantile- and juvenile-onset hypophosphatasia (HPP)
BLA 761025	ORIGINAL-1	PRAXBIND	IDARUCIZUMAB	BOEHRINGER INGELHEIM PHARMACEUTICALS INC	16-Oct-2015	Treatment of patients treated with Pradaxa® when reversal of the anticoagulant effects of dabigatran is needed for emergency surgery/urgent procedures and in life-threatening or uncontrolled bleeding

BLA 125554	SUPPLEMENT-5	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	09-Oct-2015	Treatment of patients with metastatic non-squamous non-small cell lung cancer (NSCLC) with progression on or after platinum-based chemotherapy. Patients with EGFR or ALK genomic tumor aberrations should have disease progression on FDA-approved therapy for these aberrations prior to receiving OPDIVO
BLA 125514	SUPPLEMENT-5	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	02-Oct-2015	Treatment of patients with metastatic, PD-L1 positive, non-small cell lung cancer (NSCLC), as determined by an FDA-approved test, with disease progression on or after platinum-containing chemotherapy
NDA 208169	ORIGINAL-1	XURIDEN	URIDINE TRIACETATE	WELLSTAT THERAPEUTICS CORP	04-Sep-2015	Treatment of hereditary orotic aciduria
NDA 207931	ORIGINAL-1	TECHNIVIE	OMBITASVIR, PARITAPREVIR, RITONAVIR	ABBVIE INC	24-Jul-2015	Treatment of patients with genotype 4 chronic hepatitis C virus (HCV) infection without cirrhosis
NDA 206038	ORIGINAL-1	ORKAMBI	LUMACAFTOR/IVA CAFTOR	VERTEX PHARMACEUTICALS INC	02-Jul-2015	Treatment of cystic fibrosis (CF) in patients age 12 years and older who are homozygous for the F508del mutation in the CFTR gene
NDA 21110	SUPPLEMENT-73	RAPAMUNE	SIROLIMUS	PF PRISM CV	28-May-2015	Treatment of patients with lymphangioleiomyomatosis (LAM)
NDA 21083	SUPPLEMENT-55	RAPAMUNE	SIROLIMUS	PF PRISM CV	28-May-2015	Treatment of patients with lymphangioleiomyomatosis (LAM)
BLA 125387	SUPPLEMENT-48	EYLEA	AFLIBERCEPT	REGENERON PHARMACEUTICALS INC	25-Mar-2015	Treatment of diabetic retinopathy (DR) in patients with diabetic macular edema (DME)
NDA 207925	ORIGINAL-1	KALYDECO	IVACAFTOR	VERTEX PHARMACEUTICALS INC	17-Mar-2015	Treatment of cystic fibrosis patients 2 years and older who have one of the following mutations in CFTR gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R, and R117H
BLA 125156	SUPPLEMENT-106	LUCENTIS	RANIBIZUMAB	GENENTECH INC	06-Feb-2015	Treatment of Diabetic Retinopathy (DR) in patients with Diabetic Macula Edema (DME)

NDA 207103	ORIGINAL-1	IBRANCE	PALBOCICLIB	PFIZER INC	03-Feb-2015	Treatment of postmenopausal women with estrogen receptor (ER)-positive, human epidermal growth factor receptor 2 (HER2)-negative advanced breast cancer as initial endocrine-based therapy for their metastatic disease
NDA 205552	SUPPLEMENT-2	IMBRUVICA	IBRUTINIB	PHARMACYCLICS LLC	29-Jan-2015	Treatment of patients with Waldenström's macroglobulinemia
NDA 203188	SUPPLEMENT-14	KALYDECO	IVACAFTOR	VERTEX PHARMACEUTICALS INC	29-Dec-2014	Treatment of cystic fibrosis in patients age 6 years and older who have the R117H mutation in the CF transmembrane conductance regulator (CFTR) gene
BLA 125554	ORIGINAL-1	OPDIVO	NIVOLUMAB	BRISTOL-MYERS SQUIBB COMPANY	22-Dec-2014	Treatment of unresectable or metastatic melanoma and disease progression following ipilimumab and, if BRAF V600 mutation positive, a BRAF inhibitor
NDA 206619	ORIGINAL-1	VIEKIRA PAK	OMBITASVIR, PARITAPREVIR, RITONAVIR	ABBVIE INC	19-Dec-2014	Treatment of patients with genotype 1 chronic hepatitis C virus (HCV) infection including those with compensated cirrhosis
BLA 125557	ORIGINAL-1	BLINCYTO	BLINTUMOMAB	AMGEN INC	03-Dec-2014	Treatment of Philadelphia chromosome-negative relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL)
NDA 22535	ORIGINAL-1	ESBRIET	PIRFENIDONE	GENENTECH INC	15-Oct-2014	Treatment of idiopathic pulmonary fibrosis (IPF)
NDA 205832	ORIGINAL-1	OFEV	NINTEDANIB	BOEHRINGER INGELHEIM PHARMACEUTICALS INC	15-Oct-2014	Treatment of idiopathic pulmonary fibrosis (IPF)
NDA 205834	ORIGINAL-1	HARVONI	SOFOSBUVIR/LEDI PASVIR	GILEAD SCIENCES INC	10-Oct-2014	Treatment of chronic hepatitis C, genotype 1 infection
BLA 125514	ORIGINAL-1	KEYTRUDA	PEMBROLIZUMAB	MERCK SHARP & DOHME CORP	04-Sep-2014	Treatment of patients with unresectable or metastatic melanoma & disease progression following ipilimumab and, if BRAF V600 mutation positive, a BRAF inhibitor
NDA 22291	SUPPLEMENT-12	PROMACTA	ELTROMBOPAG	GLAXOSMITHKLINE	26-Aug-2014	Treatment of cytopenias in patients with severe aplastic anemia who have had an insufficient response to immunosuppressive therapy

NDA 205552	SUPPLEMENT-1	IMBRUVICA	IBRUTINIB	PHARMACYCLICS INC	28-Jul-2014	Treatment of patients with chronic lymphocytic leukemia (CLL) who have received at least one prior therapy and CLL with 17p deletion
NDA 206545	ORIGINAL-1	ZYDELIG	IDELALISIB	GILEAD SCIENCES INC	23-Jul-2014	Treatment of relapsed chronic lymphocytic leukemia (CLL), in combination with rituximab, in patients for whom rituximab alone would be considered appropriate therapy due to other co-morbidities
NDA 205755	ORIGINAL-1	ZYKADIA	CERITINIB	NOVARTIS PHARMACEUTICALS CORP	29-Apr-2014	Treatment of patients with anaplastic lymphoma kinase (ALK)-positive metastatic non-small cell lung cancer (NSCLC) who have progressed on or are intolerant to crizotinib
BLA 125326	SUPPLEMENT-60	ARZERRA	OFATUMUMAB	GLAXO GROUP LIMITED D/B/A GLAXOSMITHKLINE	17-Apr-2014	Treatment of previously untreated patients with chronic lymphocytic leukemia (CLL) for whom fludarabine-based therapy is considered inappropriate
NDA 203188	SUPPLEMENT-4	KALYDECO	IVACAFTOR	VERTEX PHARMACEUTICALS INC	21-Feb-2014	Treatment of cystic fibrosis patients age 6 years and older who have mutations in the CFTR gene
NDA 204671	ORIGINAL-1	SOVALDI	SOFOSBUVIR	GILEAD SCIENCES INC	06-Dec-2013	Treatment of chronic hepatitis C infection
NDA 205552	ORIGINAL-1	IMBRUVICA	IBRUTINIB	PHARMACYCLICS INC	13-Nov-2013	Treatment of patients with mantle cell lymphoma (MCL)
BLA 125486	ORIGINAL-1	GAZYVA	OBINUTUZUMAB	GENENTECH INC	01-Nov-2013	Treatment of patients with previously untreated chronic lymphocytic leukemia in combination with chlorambucil

\* Breakthrough Therapy designation was enacted in the Food and Drug Administration Safety and Innovation Act on July 9, 2012. There were no approvals in CY 2012.